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United States Senate

April 4, 2022

The Honorable Patty Murray
Chair
Committee on Health, Education, Labor and Pensions
428 Senate Dirksen Office Building
Washington, D.C. 20510

The Honorable Richard Burr
Ranking Member
Committee on Health, Education, Labor and Pensions
835 Senate Hart Office Building
Washington, D.C. 20510

Dear Chair Murray and Ranking Member Burr:

As Senate hearings begin tomorrow on the U.S. Food and Drug Administration (FDA) user fee renewal, I write to urge you to have a patient-focused hearing that allows the rare disease community to testify at Senate Health, Education, Labor, and Pensions (HELP) Committee. Simply put, the user fee renewal process will not be successful if it ignores the end users of the medicines in the hearing process, or rubberstamps a proposal without patient input.

The overarching mission of the FDA user fee program is to provide a predictable and accountable regulatory framework that supports expedited FDA review and approval of pharmaceuticals and medical devices, ensuring Americans receive access to safe and effective products. Recognizing the importance of incorporating patient voices in the review and approval of medical products, the FDA, medical industries, and Congress have taken steps to encourage patient-focused drug and device development. Integrating patient perspectives with drug development is especially important for patients with rare, lesser known, progressive, and serious diseases and conditions, as well as for diseases with unmet clinical. It is therefore critical that the FDA user fee program incorporate patient experiences and perspectives, especially patients with rare diseases and unmet needs.

In September 1992, Congress passed the *Prescription Drug User Fee Act* into law, authorizing the FDA to collect fees from pharmaceutical companies to review their product applications for approval. Since its enactment, Congress has reauthorized the user fee program every five years, and is responsible for reauthorizing the program for the sixth time in September 2022. Over the last 30 years, the user fee program has expanded to include medical devices, generic drugs, and biosimilars. Importantly, the user fee program reauthorization process has

evolved into an opportunity for Congress to direct FDA and medical industries to implement new policies for the benefit of patients, including 21st Century tools and techniques to address rare disease drug development.

While the FDA, medical industries, and Congress have made great improvements to include patients and patient perspectives in the user fee program, there is still more to be done to ensure that patients with rare diseases receive representation and consideration for the September 2022 user fee reauthorization. For example, during the 2012 and 2017 user fee reauthorization process, the Senate HELP Committee invited a witness to provide the patient perspective and testimony, in addition to witnesses from the pharmaceutical and medical device trade associations, at user fee reauthorization hearings. I commend the Committee for its efforts to ensure patient representation in user fee reauthorization discussions and hearings. However, I am concerned about the lack of representation of patients with rare diseases at upcoming HELP hearings, especially given the considerable number of provisions related to rare disease drug and device development included in FDA and industry's final user fee agreements for 2022.

In general, rare and lesser known diseases present a unique set of challenges, particularly for drug and device development, clinical trial design, and patient access to therapies, that are not commonly experienced during traditional product development. For example, clinical trials for rare disease drugs often struggle to recruit and maintain a patient sample size large enough to generalize the data, as well as lack biomarkers and endpoints to adequately demonstrate efficacy. Despite increased efforts to address these issues and expedite approval of rare disease treatments, more than 90 percent of rare diseases have no treatment.[1] Unfortunately, the current regulatory framework for rare disease product development has failed to support efficient development, review, and approval of treatments for rare diseases, further preventing patients from access to therapies.

The FDA user fee program and reauthorization has helped modernize FDA regulatory requirements, establish new approval pathways, and improve the Agency and development process in tandem with medical and technological advancements to ensure expedited approval of products that benefit patients across the United States and globally. The FDA and industry final agreements reflect a shift in FDA and industry priorities to encourage and advance rare disease product development and accessibility. As a result, it is imperative that Senate ensures the rare disease community is represented by a witness in the on-going user fee reauthorization discussions held in the Senate HELP Committee to ensure the translational success of new policies incorporated into the final user fee agreement.

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[1] Nord, "New Report Finds Medical Treatments for Rare Diseases Account for Only 11% of U.S. Drug Spending; Nearly 80% of Orphan Drug Products Treat Rare Diseases Exclusively," Mar. 4, 2021, https://rarediseases.org/new-report-finds-medical-treatments-for-rare-diseases-account-for-only-11-of-us-drug-spending-nearly-80-of-orphan-products-treat-rare-diseases-exclusively/#:~:text=Approximately%207%2C000%20known%20rare%20diseases,rare%20diseases%20have%20no%20treatment.

For these reasons, I request that the Senate HELP Committee provide a full patient representative hearing from the rare and life threatening disease community in order to advance medical product regulation and innovation for the benefit of patients, including those diagnosed with rare diseases. I appreciate your attention to this request, and my staff and I are willing to help you both organize this hearing.

Sincerely,

Mike Braun

Senator Mike Braun